

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property Organization
International Bureau(43) International Publication Date
25 October 2001 (25.10.2001)

PCT

(10) International Publication Number
WO 01/79283 A1(51) International Patent Classification: C07K 14/47,
C12N 15/87, 5/02, A61K 38/17, 31/7052, A61P 25/28,
15/08, A61K 39/395(74) Agent: MBM & CO.; P.O. Box 809, Station B, Ottawa,
Ontario K1P 5P9 (CA).

(21) International Application Number: PCT/CA01/00495

(22) International Filing Date: 12 April 2001 (12.04.2001)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
2,305,088 13 April 2000 (13.04.2000) CA
2,326,543 12 December 2000 (12.12.2000) CA(71) Applicant (for all designated States except US): UNIVER-
SITY OF BRITISH COLUMBIA [CA/CA]; Industry Li-
aision Office, 111-2386 East Mall, Vancouver, British Co-
lumbia V6T 1Z3 (CA).

(72) Inventors; and

(75) Inventors/Applicants (for US only): HAYDEN, Michael,
R. [CA/CA]; 4484 West 7th Avenue, Vancouver, British
Columbia V6R 1W9 (CA). HACKAM, Abigail, S.
[CA/US]; 105 West 39th St., Apt. 1219, Baltimore, MD
21210 (US). LEAVITT, Blair, R. [CA/CA]; 4008 West
32nd Ave., Vancouver, British Columbia V6S 1Z6 (CA).(81) Designated States (national): AE, AG, AL, AM, AT, AU,
AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU,
CZ, DE, DK, DM, DZ, EE, ES, FI, GB, GD, GE, GH, GM,
HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK,
LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX,
MZ, NO, NZ, PL, PT, RO, RU, SD, SE, SG, SI, SK, SL,
TJ, TM, TR, TT, TZ, UA, UG, US, UZ, VN, YU, ZA, ZW.(84) Designated States (regional): ARIPO patent (GH, GM,
KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZW), Eurasian
patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European
patent (AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE,
IT, LU, MC, NL, PT, SE, TR), OAPI patent (BF, BJ, CF,
CG, CI, CM, GA, GN, GW, ML, MR, NE, SN, TD, TG).

Published:

- with international search report
- before the expiration of the time limit for amending the
claims and to be republished in the event of receipt of
amendments

For two-letter codes and other abbreviations, refer to the "Guide-
ance Notes on Codes and Abbreviations" appearing at the begin-
ning of each regular issue of the PCT Gazette.



WO 01/79283 A1

(54) Title: MODULATING CELL SURVIVAL BY MODULATING HUNTINGTIN FUNCTION

(57) Abstract: The present invention provides a means of modulating cell survival by modulating wild-type huntingtin protein function by administration of wild-type huntingtin protein, biologically active fragments thereof and/or antagonists of wild-type huntingtin. Accordingly, the present invention also provides biologically active fragments of wild-type huntingtin, antagonists of wild-type huntingtin and nucleic acid sequences encoding the biologically active fragments and peptide antagonists, and their use to modulate cell survival. In particular, the invention provides for means to activate or attenuate cell death within tissue, in order to facilitate the treatment of conditions where there is a dysregulation of cell death or cellular proliferation. Therapeutic application of this invention pertains to diseases and disorders including, but not limited to, Huntington disease, neurodegenerative diseases, stroke, and cancer.